BMS Response to HTA Review Options Paper:

The HTA review represents a significant opportunity to i) elevate the patient voice in HTA decision making, ii) improve timeliness of access to medicines for Australian patients, and iii) future proof the system to allow for the inevitable advances that science and medicine will deliver.

Bristol Myers Squibb Australia (BMSA) believes that the options outlined in the HTA review paper specific to enhanced consumer engagement and horizon scanning are needed, well thought out and will deliver upon opportunities i) & iii) outlined above.

However, BMSA sees the options outlined specific to streamlined pathways as exacerbating rather than addressing the current access issues in Australia. BMSA is wholly opposed to any proposed measures relating to changes to current pricing policy in the options paper. Proposals to deliver savings to the commonwealth through pricing initiatives are outside the terms of reference of the HTA Review and should be rejeted. If recommended and agreed by the Minister of Health and Ageing, the launch of new medicines and treatments in Australia will be significantly delayed or not occur at all.

All stakeholders participating in the HTA review acknowledge that the time taken between TGA registration and PBS listing in Australia is far too long and needs to be improved. Yet the options paper only puts forward potential changes relevant to transformative medicines in areas of HUCN *(~<5% of submissions)* and medicines seeking listing via the cost-minimisation pathway *(~30% of submissions – cost-min)*. The paper does not have any concrete proposals regarding the single largest group of medicines that contribute to the time gap – i.e. those medicines that advance current treatment algorithms by delivering superior health outcomes *(~65% of submisisons – cost-effectiveness)*. Rather, there is a vague proposal to asses the pilot for HUCN submissions and then potentially expand this to relevant cost effectiveness submissions at some indeterminate time in the future.

BMSA implores the Committee to recommend pathway changes specific to ***all*** PBAC submissions, thereby genuinely addressing the intent of the HTA review – to reduce the time between TGA registration and PBS listing. In order to generate workable recommendations, two of the central key tenets of HTA, namely value and uncertainty, need to be addressed and factored into the proposed pathway changes.

Please find below more fulsome feedback on the specific options outlined in Chapters 1 through 5 of the HTA Review Options Paper.

Chapter 1: Transparency, communication, and stakeholder involvement in HTA

1.1 Transparency and Communication of HTA pathways, processes and decisions

BMSA supports the implementation of plain language summaries for PBAC submissions. BMSA participated in the Summary of Information pilot and actively supports efforts to ensure patient organisations, and in turn patients, have information that facilitates their ability to provide input into HTA decision making and further, to support robust decision making.

BMSA believes that criteria are needed to define which submissions are appropriate and would benefit from formal summaries and does not advoate that all submissions require a summary.

In order for summaries and discourse with patient organisations at the time of PBAC submissions to be possible, BMSA encourages the Committee to have regard for the current legislative barriers including the direct to consumer guidance. Greater clarity around what is promotional or the legislative change required to facilitate early consumer engagement is critical.

Plain language summaries of decisions would be welcomed by BMSA and other stakeholders and this has been relfected in our consultation with patient organisations and described in the following reports prepared by Biointelect:

- Broadening the Evidence[1]
- Bringing Patient Centricity to Life System Reform[2]

BMSA acknowledges the capacity gaps that exist for many stakeholders and therefore also supports the development of educative resources and information related to HTA pathways and PBAC guidelines as well as the development of platforms that support access to information (via the HTA webpage dashboard).

1.2 Consumer, clinician and other stakeholder engagement and consideration in HTA

BMSA supports the **development of an engagement framework** that faciltates earlier and consistent engagement of consumers and clinicians throughout HTA processes.

Again, BMSA encourages the Committee to consider any changes or updates to legislation that allow for greater discourse between industry and consumers earlier in HTA processes.

BMSA supports the option for comparator and outcome (PICO) workshops to ensure that correct key indicators that reflect the Australian context are being considered. We note that legislation requiring the choice of comparator would need to be amended.

With regards to items that are being removed from the PBS, we support a transparent, robust approach that ensures Australian patients are not adversely affected and are provided with as much information to prepare for any changes as possible.

BMSA supports the option to **strengthen consumer evidence** and the development of curated lists/tools that provide guidance on how to best collect data from patients and the community. BMSA supports the generation of data that aids robust PBAC decision making that priortises patient needs, values and preferences.

1.3 First nations people involvement and consideration in HTA

BMSA supports all closing the gap initiatives and recognises the tremendous issues facing Indigenous Australians' healthcare. BMSA would encourage the Committee to consider criteria for sponsor submissions requiring considerations and assessment of impact for First Nations people. Noting however, the ability to impact clinical trial protocols is limited for local affiliates of multi-national companies, and as such, applicable data may be limited.

1.4 State and Territory Collaboration in HTA

BMSA supports in principle the options presented to address issues relating to the HTA assessment and provision of highly specialised therapies (HSTs) that may be jointly funded by the commonwealth and the states and territories.

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[1] https://www.bms.com/assets/bms/australia/documents/Broadening-the-evidence.pdf

[2] https://www.bms.com/assets/bms/australia/documents/Bringing%20Patient%20Centricity%20to%20Life%20in%20Australian%20Healthcare.pdf

We encourage all jurisdictions to complete the work identified in Schedule C of the Addendum to National Health Reform Agreement 2020-25 to implement "*a financing system that is proactive, value-based and focused on individual and community needs*"[3] as soon as practicable. This should support a framework for the appropriate funding of HSTs and clarify the costs for all parties. A full, transparent and agreed understanding of the costs for jurisdictions will reduce delays in access by patients to HSTs.

BMSA recognises the important role horizon scanning can play in helping jurisdictions prepare for the introduction of innovative medicines, particularly from a budget perspective. We support the options proposed in this section and in 5.2 of the options paper.

Any new national approach to the assessment and funding of innovative medicines must maintain equity of access for patients. In particular, the options relating to consultation, data and work sharing must not delay unreasonably patient access to treatment.

Chapter 2: Health technology funding and assessment pathways

2.2 Proportionate appraisal pathways

BMSA acknowledges the proposed options for '*early resolution mechanisms for submissions of major new therapeutic advances in areas of HUCN'*, however wishes to note that:

- these options look to formalize processes that currently occur[4], and as such, will not deliver significant improvement in the time to PBS listing for the ~<5% of submissions classified as delivering therapeutic advances in areas of HUCN, and
- the potential to expand the resolution step to all cost-effectiveness submissions (~65% of PBAC submissions) is only hinted at in the longer term – i.e. '*after piloting with therapies in HATV in areas of HUCN the early resolution step <u>could</u> be expanded to other <u>relevant</u> cost-effectiveness submissions'*.

BMSA believes that the options outlined in Chapter 2.2 specific to early resolution need to be expanded to ***all*** cost-effectiveness submissions in order to improve timely access to medicines for Australian patients. Gaining as much alignment as possible on the decision problem and analytical approach at an early stage of the HTA process will be more efficient than addressing these issues via re-submissions. BMSA believes that a target of 100 days from TGA registration to PBS listing should be the aim for applications where clinical outcomes are improved (ie cost-effectiveness submission) – and that a system set up for early dialogue and agreement across all stakeholders on value and sharing of uncertainty would deliver on such an aim. Improvements with regards to transparency and timelines within the post PBAC process would also assist with delivering on a target of 100 days from TGA registration to PBS listing.

Chapter 3: Methods for HTA for Australian Government Subsidy (technical methods)

3.2 Clinical evaluation methods

BMSA acknowledges the proposed options to review many of the technical methods used in HTA in Australia – including the need to revise and update guidance specific to the use of indirect treatment

[3] Addendum to National Health Reform Agreement 2020-25, chrome-extension://efaidnbmnnnibpcajpcglclefindmkaj/https://federalfinancialrelations.gov.au/sites/federalfinancialrelations.gov.au/files/2021-07/NHRA_2020-25_Addendum_consolidated.pdf

[4] e.g. Opdivo + Yervoy for the treatment of mesothelioma – 50 days between TGA and PBS

comparison (ITC), non-randomised studies, real world evidence (RWE), surrogate outcomes, value assessment and dealing with uncertainty in HTA submissions.

BMSA supports the need for revision of technical methods and updated guidance and in doing so, requests that all stakeholders are involved in finalizing the changes and that they are implemented as a priority.

3.3 Economic evaluation – comparator

One area where significant work and alignment is needed immediately relates to the definition and use of the main comparator in PBAC submissions. Currently there is a disconnect between industry and the PBAC with regards to the definition and use of the main comparator.

BMSA supports the position put by our industry body, Medicines Australia, that options relating to comparator selection should be significantly strengthened. In particular, to align with global HTA comparator selection, which is to select the therapy most likely to be replaced in practice.

One of the key objectives of the HTA Review is to identify features of HTA which *"may act as current or future barriers to earliest possible access"*. BMSA would contend that failure to recognise the value of an innovative medicines by comparing them to the lowest cost comparator in HTA is a clear barrier to early access as it acts as a disincentive to bringing these medicines to patients in Australia.

BMSA recognises that the current interpretation of Section 101 (3B) of the national Health Act by the PBAC gives rise to this potential access delay. This could be resolved by better defining what "*alternative therapy*" means in the National Health Act. Most simply, section 101 (3B) could be amended to better define alternative therapy as "*the treatment that is most likely to be replaced in clinical practice*". We note that Medicines Australia has also proposed this as an option, along with other alternatives and BMSA urges the review committee to consider these options.

Ensuring the value of innovation and true sharing of clinical, economic and financial uncertainty are recognised as key elements that are required within any new pathways defined to reduce the timeline between TGA registration and PBS listing. These factors need to incorporated within pathway recommendations to the Minister of Health and Ageing.

Chapter 4: Health technology funding and purchasing approaches and managing uncertainty

4.1 Approaches to funding or purchasing new health technologies

BMSA believes that the options put forward with regards to *recognizing competition between new health technologies that deliver similar outcomes* has the potential to not only exacerbate the time to access issues we currently have in Australia, but to also see global pharmaceutical organisations de-prioritise Australia as a first-wave launch country.

Global organisations consistently challenge their Australian affiliates with regards to the low net pricing in Australia compared to other developed countries. Pricing certainty within F1 once listed on the PBS is the major contributor to keeping Australia within first-wave launch countries. BMSA sees the options detailed in Chapter 4 as reducing this pricing certainty for both cost-minimisation and cost-effectiveness medicines, and as such risks Australia being de-prioritised with regards to launching new medicines.

Cost-Minimisation Submissions:

The current PBAC guidelines and HTA system work well where clinical trials exist that directly compare the new medicine versus the current standard of care (Main Comparator). However, the PBAC guidelines and HTA system present significant challenges in i) proving superior clinical and cost-

effectiveness via indirect treatment comparison (ITC) & ii) proving clinical and cost-effectiveness in disease areas with small patient populations.

Whilst the PBAC guidelines allow the use of ITCs, a clinical claim of superiority based upon ITC is very rarely agreed by the PBAC. Potential transitivity issues are used to over-index potential uncertainty with regards to the clinical and cost-effectiveness comparison being presented - which leads to PBAC rejection/s (delay) and/ or potential under-valuation of the new medicine.

In addition, potential clinically relevant endpoints associated with safety and compliance are not valued under the current HTA construct/ PBAC guidelines. Quite often new medicines show advantages over current standard of care with respect to safety and compliance endpoints. Yet as they have not been powered to show a statistically significant difference in clinical trials, they are very rarely able to be incorporated in determining the value of the new medicine.

Due to above stringent evidentiary standards, options detailed in Chapter 4 present the issue that medicines with potential improved patient outcomes versus the main comparator will only be listed on the PBS if they are priced lower than the main comparator. This will clearly present significant challenges when seeking approval from global affiliates to proceed with launching these medicines in Australia.

Cost-Effectiveness Submissions:

Options in Chapter 4 specific to the pricing of medicines via the cost-minimisation pathway also risks the delay or non-launch of medicines that deliver significantly improved health outcomes versus the current standard of care.

The cost-effectiveness of a new medicine is determined by comparing the efficacy, safety and pricing of the new medicine versus the main comparator (standard of care). The options put forward in Chapter 4, combined with reference pricing policies, would see an ever diminishing price for the main comparator, thereby making it more difficult for new breakthrough medicines to prove cost-effectiveness. As a result, global organisations will de-prioritise Australia as a launch country.

As such, BMSA supports Medicines Australia's view with regards to options provided in Chapter 4 – i.e. "*Medicines Australia strongly opposes this option as it does not solve any identified issues and it is outside the terms of reference for the Review. Moreover, it will have serious negative consequences for patients. It is in patients' best interests to have access to a range of treatment options, ensure diversity of supply and minimise the risk of products leaving the market (or not being launched at all) due to unacceptable post-launch pricing implications. If two medicines confer similar benefit there is no HTA-based reason to justify a lower price. The proposal of incentivising or requiring Sponsors to propose further discounts is a departure from the intent of the HTA review because it will ultimately prevent products coming to market and lead to unviable price erosion post-launch. There are already many existing price controls including statutory price reductions, reference pricing and post-market reviews amongst other administrative price reductions.*"

BMSA also supports the position of Medicines Australia that the post-listing reassessment of medical technologies recommendation should not appear in the report. Options for disinvestment as recommended by the review committee do nothing to appropriately value medicines or speed up access for patients. The post-market assessment process, while somewhat slow and cumbersome, does an adequate job of assessing technologies after listing on the PBS and has recently been updated in consultation with industry and other stakeholders.

Chapter 5: Futureproofing Australia's systems and processes

5.1 Proactively addressing areas of unmet clinical needs and gaps in the PBS

BMSA supports options that facilitate greater preparedness for all stakeholders with regards to medicines that may impact upon the lives of Australian patients. Moreover, BMSA supports **proactively addressing areas on unmet clinical need and gaps in the PBS.**

We strongly encourage the Committee to ensure that recommendations around future proofing Australia's systems and processes include the involvement of industry stakeholders as well as consumers, clinicians and institutional representation and others. The goal should be to reduce the time to access for medicines reaching Australian patients as a result of horizon scanning and system preparedness.

5.2 Establishment of horizon scanning programs

BMSA proposes that an independent body should govern horizon scanning processes.

5.6 Strengthen international partnership and work-sharing

BMSA, while not opposed to some of the benefits that might arise from greater international collaboration, is opposed to the option that recommends investigating "*opportunities for collaboration with international jurisdictions to increase market share and purchasing power for innovative health technologies which address areas of HUCN*". Australia already pays some of the lowest prices in the developed world for innovative medicines. It is hard to see how this initiative would improve matters in terms of appropriate valuation of innovation and speed of access by patients.